



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2020-N-0026]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the applicant of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (FD&C Act) authorizes FDA to award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA is required to publish notice of the award of the priority review voucher. FDA has determined that SKYSONA (elivaldogene autotemcel), manufactured by bluebird bio, Inc., meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Myrna Hanna, Center for Biologics

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SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the applicant of an approved rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), FDA will award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA has determined that SKYSONA (elivaldogene autotemcel), manufactured by bluebird bio, Inc., meets the criteria for a priority review voucher. SKYSONA is indicated to slow the progression of neurologic dysfunction in boys 4 to 17 years of age with early active cerebral adrenoleukodystrophy.

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <https://www.fda.gov/industry/developing-products-rare-diseases-conditions/rare-pediatric-disease-rpd-designation-and-voucher-programs>. For further information about SKYSONA, go to the Center for Biologics Evaluation and Research Cellular and Gene Therapy Products website at <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products>.

Dated: September 30, 2022.

Lauren K. Roth,

Associate Commissioner for Policy.

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